September 23, 2019

Abeona Therapeutics Provides Regulatory Update Ahead of Pivotal Phase 3 Clinical Trial for EB-101 in Recessive Dystrophic Epidermolysis Bullosa

- Company to provide additional transport stability data points in response to FDA Clinical Hold Letter
- CMC clearance for VIITAL™ trial anticipated in Q4 2019
- Investor conference call today, Monday, September 23 at 09:30 a.m. ET

NEW YORK and CLEVELAND, Sept. 23, 2019 (GLOBE NEWSWIRE) -- Abeona Therapeutics Inc. (Nasdaq: ABEO), a fully-integrated leader in gene and cell therapy, today announced that it has recently received a clinical hold letter from the U.S. Food and Drug Administration (FDA) clarifying that the FDA will not provide approval for the Company to begin its planned Phase 3 clinical trial for EB-101 until it submits to the FDA additional data points on transport stability of EB-101 to clinical sites. Over the last 12 months, the Company has worked closely with the FDA to address and narrow open Chemical, Manufacturing and Controls (CMC) items and has been working to resolve this one item identified in the FDA Clinical Hold Letter. The Company continues to anticipate receiving CMC clearance for VIITAL™ trial in Q4 2019.

“Initiating the VIITAL™ pivotal Phase 3 trial for EB-101 is the Company’s top priority,” said João Siffert, M.D., Chief Executive Officer of Abeona. “Efforts to gather supplemental data points on transport stability of EB-101 are ongoing and we are confident that the requested additional data will be submitted to the FDA promptly. Looking ahead, we believe that completion of our CMC work coupled with the durable safety and efficacy data, now out to five years in some patients, will ultimately be critical to support a future Biologics License Application. We remain deeply committed to advancing EB-101 to provide a desperately needed treatment for RDEB patients.”

The Company will hold a conference call scheduled for Monday, September 23 at 09:30 a.m. ET. Interested parties are invited to participate in the call by dialing 844-369-8770 (toll-free domestic) or 862-298-0840 (international) or via webcast at https://www.investornetwork.com/event/presentation/53728.

About EB-101
EB-101 is an investigational, autologous, gene-corrected cell therapy poised to enter late-stage development for the treatment of recessive dystrophic epidermolysis bullosa (RDEB), a rare connective tissue disorder without an approved therapy. Treatment with EB-101 involves using gene transfer to deliver COL7A1 genes into a patient’s own skin cells (keratinocytes) and transplanting them back to the patient to enable normal Type VII

collagen expression and facilitate wound healing. In the U.S., Abeona holds Regenerative Medicine Advanced Therapy, Breakthrough Therapy, and Rare Pediatric designations for EB-101 and Orphan Drug designation in both the U.S. and EU.

About Recessive Dystrophic Epidermolysis Bullosa
Recessive dystrophic epidermolysis bullosa, or RDEB, is a rare connective tissue disorder characterized by severe skin wounds that cause pain and can lead to systemic complications impacting the length and quality of life. People with RDEB have a defect in the COL7A1 gene, leaving them unable to produce functioning Type VII collagen which is necessary to anchor the dermal and epidermal layers of the skin. There is currently no approved treatment for RDEB.

About Abeona Therapeutics
Abeona Therapeutics Inc. is a clinical-stage biopharmaceutical company developing gene and cell therapies for serious diseases. The Company's clinical programs include EB-101, its autologous, gene-corrected cell therapy for recessive dystrophic epidermolysis bullosa, as well as ABO-102 and ABO-101, novel AAV9-based gene therapies for Sanfilippo syndrome types A and B (MPS IIIA and MPS IIIB), respectively. The Company's portfolio of AAV9-based gene therapies also features ABO-202 and ABO-201 for CLN1 disease and CLN3 disease, respectively. Its preclinical assets include ABO-401, which uses the novel AIM™ AAV vector platform to address all mutations of cystic fibrosis. Abeona has received numerous regulatory designations from the FDA and EMA for its pipeline candidates and is the only company with Regenerative Medicine Advanced Therapy designation for two candidates (EB-101 and ABO-102). For more information, visit www.abeonatherapeutics.com.

Forward Looking Statement
This press release contains certain statements that are forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, and that involve risks and uncertainties. These statements include statements about the timing for CMC clearance for the VIITAL™ trial and the Company's beliefs relating thereto, the Company's ability to provide additional transport stability data points in response to the FDA clinical hold letter and the timing thereof, the Company's belief that completion of its CMC work and the durable safety and efficacy data will ultimately be critical to support a future Biologics License Application, the ability of its management team to lead the Company and deliver on key strategies, the market opportunities for the Company's products and product candidates, and the Company's goals and objectives. We have attempted to identify forward looking statements by such terminology as "may," "will," "anticipate," "believe," "estimate," "expect," "intend," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances), which constitute and are intended to identify forward-looking statements. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, numerous risks and uncertainties, including but not limited to continued interest in our rare disease portfolio, our ability to enroll patients in clinical trials, the impact of competition, the ability to secure licenses for any technology that may be necessary to commercialize our products, the ability to achieve or obtain necessary regulatory approvals, the risk of whether or when the FDA will lift the clinical hold respecting the Company's planned Phase 3 clinical trial for EB-101, the impact of changes in the financial markets and global economic conditions, risks associated with data analysis and

reporting, and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and quarterly reports on Form 10-Q and other reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligation to revise the forward-looking statements or to update them to reflect events or circumstances occurring after the date of this presentation, whether as a result of new information, future developments or otherwise, except as required by the federal securities laws.

Investor Contact:
Sofia Warner
Senior Director, Investor Relations
Abeona Therapeutics
+1 (646) 813-4710
swarner@abeonatherapeutics.com

Media Contact:
Scott Santiamo
Director, Corporate Communications
Abeona Therapeutics
+1 (718) 344-5843
ssantiamo@abeonatherapeutics.com

Source: Abeona Therapeutics Inc.